

LETTER

A preliminary analysis of luspatercept use and expenditure in Italy in the first semester of 2022

To the Editor,

We read with interest the article by Pilunni and Navarra [1], in which the authors analysed compassionate use programs (CUPs) in Italy reporting data on the use of twelve different drugs including luspatercept (Reblozyl®). It is a recombinant fusion protein that stimulates the production of erythrocytes allowing late-stage erythroblast differentiation and erythroid maturation [2] and represents a potential treatment for adult patients with transfusion-dependent myelodysplastic syndromes [3] or β -thalassaemia [4]. On November 2021, a marketing authorization was issued for luspatercept by the Italian Medicines Agency (Agenzia Italiana del Farmaco, AIFA) and reimbursed by the National Health System [5] for the treatment of adult patients with transfusion-dependent anaemia caused by very low, low, and intermediate-risk myelodysplastic syndromes with ring sideroblasts (MDS-RS) who had an unsatisfactory response to or are ineligible for erythropoietin-based therapy, or those with transfusion-dependent beta thalassaemia (BT). Luspatercept is available as powder for solution for injection containing 25 mg/vial or 75 mg/vial. The recommended starting dose is 1.0 mg/kg administered once every 3 weeks and it could be increased up to 1.75 mg/kg in non-respondent patients.

In order to evaluate the national consumption of luspatercept in the first semester of 2022, we conducted a preliminary analysis using National Observatory on the Use of Medicinals (OsMed) database of the AIFA, which collects data of the purchase by public health facilities. Moreover, public expenditure, compounded monthly growth rate (CMGR), number of treated patient and cost per patients were estimated.

From January to June 2022, 5,248 packages of luspatercept have been provided in Italy for treatment of targeted patients (2,403 units of 25 mg/vial and 2,845 of 75 mg/vial). The total expenditure was about 8.9 million euros (about 1.9 million euros for 25 mg/vial and about 7 million euros for 75 mg/vial). The CMGR was +37% (from 353,469 euros on January to 2,291,899 euros on June 2022); the steady increase of costs reflects the fact that the various Italian Regions/Autonomous Provinces progressively included luspatercept in the list of drugs that can be purchased by public health facilities. Based on the schedule of administration of luspatercept and the period of utilisation in each Region/Autonomous Province, we estimated that 424 patients were treated with an average increase of 62 patients per month. Moreover, using expenditure data from the OsMed database and the estimated patient number, a cost of 20,800 euros per patient was also estimated. Our estimations are consistent with recent published data on the luspatercept [1] that seems to fill a long-standing gap by providing a much-needed additional treatment option for a subset of adult patients with transfusion-dependent anaemia reducing transfusion burden, transfusion complications and use of iron-chelating agents. At the moment, the medical conditions in which luspatercept can be used for are limited but some studies are investigating its possible use in other haematological and non-haematological diseases with a potential enlargement of the patients who could benefit of this pharmacological treatment. Therefore, it is crucial to continue to collect data about its efficacy and safety in a real-world practice and our analysis, aimed to evaluate the luspatercept use in the first post-authorization semester, represents a starting point in order to perform permanent monitoring of its clinical and economic burden.

Key words

- anemia
- luspatercept
- drug use
- drug expenditure

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